NEWSLETTER

HUNTINGTONS QUEENSLAND

March 2013

CONTENTS

FROM THE PRESIDENT

Dear Friends

This year has started with very good news on the fund raising front. We have been successful in obtaining a grant from the Jupiters Casino Community Benefit Fund to replace our two Toyota Camry vehicles. The other success was from The John Villiers Trust which has provided funding to replace our photocopier. Its replacement will provide not only a networked copier but a scanning and printing system for all of our operations. I would like to thank both the Jupiters Casino Community Benefit Fund and The John Villiers Trust as this has enabled Huntingtons Queensland to replace two of our key assets this year. With the uncertainty of other Government funding we are also focusing on funding for other welfare services. I would like to express my thanks on behalf of all members for the work of Cheryl, Anne and Barb Gray for their work in the preparation of these applications. We have had many other very generous donations and these are recognised later in this Newsletter.

Page 5 of this Newsletter includes the position paper on the National Disability Insurance Scheme (NDIS) developed by the Neurological Alliance Australia (NAA). Most of you would know what the NDIS is; but would not have heard about the NAA. When you look at Australia wide issues, Huntington's Disease is a small part of the national disability sector. The Neurological Alliance Australia was formed to create a much larger voice for those affected by degenerative neurological disorders such as Parkinson's, MS, MND, Alzheimer's, Fredericks Ataxia and other similar diseases. At a national level we are a member of the NAA as together we can ensure our voice is heard much more loudly.

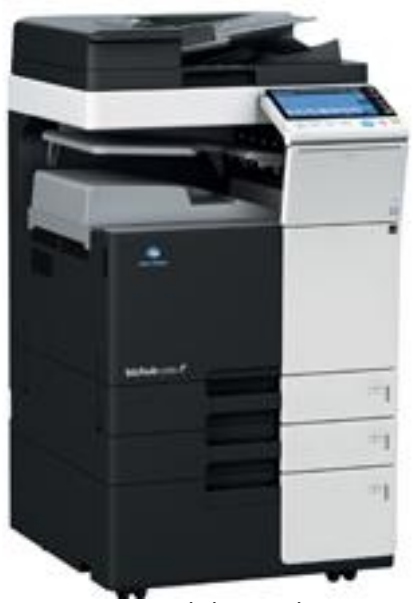
On a lighter note you may recall that last year our home, Florence Dannell House was infested by West Indian termites – the eradication treatment was done by the State Government at their cost late last year. We have just had a builder in to determine the extent of damage to the timbers in our building. We also have some water damage to check above the stairwell and whilst on the roof, the builder found a dead possum in a gutter but was also startled by a live possum "either Woody Possum or Florence Possum". If anyone wants a pet HD possum please let us know. To check the extent of the termite damage we had the panels above fire place removed which has exposed the original brick chimney. Once we know the cost of repairs we will be looking for funding to make good the damage above the fireplace and to repair the roof leak. We would welcome donations toward these building repairs.

As the financial climate continues to be fickle, we are looking forward to a very challenging but hopefully prosperous and productive year at Huntingtons Queensland.

Gerry Doyle, President

FROM THE EXECUTIVE OFFICER

Christmas has gone and New Year celebrations have past but along with January and February has come that water from the sky – rain, rain and more rain. I hope that everyone is safe and that none of our families and friends have been affected by the recent weather events.



We sure are moving along here at Annerley. The office is abuzz with technical people with their jargon flowing. Just to keep you abreast of the fun we are having; thanks to funding from The John Villiers Foundation we can replace our very old and outmoded photo copier and our individual desk printer/scanner/copiers with a very modern networked and integrated printer, scanner and copier linked to what will be our new computers. This will not only update our technology but will actually be a cost saving as the individual printers are extremely expensive to run with the cost of consumables etc. This is why we have the technical teams here with the installation of the network system and the new machines happening early March.

We are also in the process of upgrading our mobile telephones and our telephone contracts to give us a more streamlined and less expensive phone system – no number changes just a better system and new phones.

Since our last Newsletter I have met with many of the people who attend support groups and those who come to Tuesday Day Centre – AND I am even remembering names.

It is amazing what can happen in six months – my anniversary is looming.

Again we had a family holiday at Hervey Bay which from reports from Christine, Theressa and many who attended was a great success. Our thanks go to Christine and Theressa for hosting yet another great family holiday. Parents had a chance to "chill out" around the BBQ, the kids (and "big kids", mums and dads) went on a number of outings, fished, swam and generally enjoyed three days of what I am told was a "wonderful experience" and not "are we there yet?" but "when can we go again?"

Many businesses in Hervey Bay - Pialba contributed to the holiday: Coles, Woolworths, Subway, IGA, Dominos Pizza, Hervey Bay RSL and last but not least our accommodation provider Emeraldene ECO Lodge; plus donations from Sullivan and Nicolaides, Toowoomba City Golf Club and Sunnybank Community and Sports Club – a massive "thank you" to all of these organisations from us at Huntingtons Queensland and our families.

The staff here are continually considering ways in which we can best support carers and people with HD and would always be grateful for any suggestions or ideas you may have about your supporting group.

2013, the year of the snake is now well upon us. There are many different traditions observed over the Chinese New Year period and this is one of my favourites:

"Clean your house before New Year to get rid of any bad luck from the previous year and to make room for good luck".

I hope all your homes are ready for the good luck of 2013!

Cheryl Miller, Executive Officer

FROM THE WELFARE DESK…

December once again saw Christine and Theressa facilitate the annual family holiday in Hervey Bay. This year 35 people attended from around the state. There was a mix of families who have participated in the past and a number of new families who brought a new energy and dynamic to the group. As we have in previous years, we stayed at Emeraldene Lodge where Rob supports Huntingtons Queensland in a myriad of ways both large and small. He ensures that the stay for everyone is memorable and enjoyable.

After three years, the programme for the families is a mix of leisure and activity which seemed to work particularly well for the families involved this year. On the first day families met and mingled and broke the ice with each other over a barbecue. The next day was a busy day which involved free fun at Wetside. The younger children love it as do those older children (adults too) who enjoy the wave surfer. There were those amongst the group who mastered the art of hanging five with great aplomb.

We went to the newly opened Fraser Coast Discovery Sphere as a group after a delicious lunch provided by Subway, which made the lunch time catering frenzy much simpler and more manageable for everyone. The Discovery Sphere was a terrific experience for both children and adults and was the ideal "ice-breaker" for everyone. It is an interactive centre about humpback whales and the Fraser Coast. Interactive activities encouraged everybody to work together and foster communication with each other. Monday saw a night out at the Hervey Bay RSL with a delicious three course meal. Both adults and kids really seemed to enjoy the food and company as they got to know each other.

We had planned a breakfast on the beach the next morning as we had in other years, which families had particularly enjoyed, but the party atmosphere which continued after arriving back at Emeraldene ensured that we were all a little late getting the party started the next morning. Instead, one of the Dads who showed his prowess around a set of tongs on previous occasions, delivered us a beautiful poolside brunch. Afterwards everyone was very much in the mood for relaxation and the chance to fish or swim and generally just enjoy the warm weather.

These holidays are always a wonderful chance for families to share their experiences of living with HD if they choose to do that, or equally, just enjoy spending some fun time as a family, or simply hang out with other kids and enjoy doing the things kids do. Whilst all of us here at Huntingtons believe that the family holiday is a particularly rewarding and valuable experience, it's probably best said by one of the young people who attended and said to his Mum, "I want to go again – it was really good to see Dad having fun. Both you and Dad seemed happier and I liked hanging out with J and K and doing our own thing."

The Day Centre programme continues into its second decade of operation, continuing the wonderful work that previous facilitators did in getting it set up and established. The format of the programme continues largely unchanged from previous years, where we operate three Tuesdays a month, with a distinct activity on each of those days. We offer bowling the second Tuesday of the month, an outing on the third and a music therapist, Mark, comes to entertain everyone on the fourth. He comes to us after a long and distinguished career in the music industry and we love hearing where his current adventures as part of a very well known barber shop quartet have taken him. We hear of a certain mining magnate and talk of a large ship that has been one of his more recent overseas jaunts.

We have recently tried to add more outings to the programme, which because of resource constraints are relatively simple, but seem to have been well received by those attending. Last month we went to the new RSPCA Headquarters,

as a number of our group are very keen animal lovers. The outing sparked many conversations and reminiscences from our group. We have also in the past had various outings to the museum and art gallery, a trip through the newly opened tunnels followed by a picnic in a north side park and lunch along the river at New Farm Park. This month we hope to visit one of Brisbane's historic houses, and in future months when we have more predictable weather we'll incorporate outings to various well known parks for some sunshine and exercise. Feel free to pass on any places of interest to us that we might be able to access as a group.

We continue to incorporate exercises into the routine of the Day Centre, which we're always hopeful people may do on a regular basis at home, particularly in light of recent research conducted by Dr Jennifer Thompson who addressed our HD conference at The University of Queensland in September 2010. We also have regular games of Scrabble, general knowledge quizzes and games of cards, which continue to be popular with the group. Thank you to our regular volunteers who continue to support and assist us in all kinds of ways with the operation of the Day Centre and enable us to continue to provide this service.

Christine Fox (Senior Welfare Officer) along with Theressa Byrne and Fiona Kerr (Welfare Officers)

FROM THE TOWNSVILLE SUPPORT GROUP

I would like to say how great it was to see so many come along to the Townsville outings last year.

These kinds of outings allow us to get to know one another and also to share our experiences of living and loving with Huntington's in our lives. There are no right or wrong answers but by sharing how we have overcome some of life's hurdles we may be able to help each other.

It is for this reason that we have set some dates aside for the year to come together socially. We thought if we set the dates out now, we will be sure to get together as time goes by so fast. Before we know it we will be at Christmas and it will help us all plan. Townsville dates:

March 27th	2.00 pm	Afternoon tea at Queens Gardens (Kennedy St playground entrance.)
April 24th	11.30 am	The Strand (near Surf Life Saving Club Strand Park)
May 29th	10.00 am	Riverway (next to boat ramp)
June 26th	2.00 pm	Pallarenda Park
July 31st	12.00 noon	Lunch at Kirwan Tavern
August 28th	10.00 am	The Strand (near Surf Life Saving Club Strand Park)
September 25th	2.00 pm	Pallarenda Park
October 30th	11.30 am	Riverway (next to boat ramp)
November 27th	12.30 pm	Pallarenda Park

If you know of something happening around Townsville and think it may be a good place to visit please ring any of the members of the Support Group and we will look into whether we are able to get interested people together to go.

We will send out reminders closer to the date for each event but hope by knowing in advance you are able to put these events on your calendar. We look forward to seeing you in the near future.

Sue Bourne

Contacts for Townsville Support Group:

Chairperson	Sue Bourne	4778 2495	Secretary	Bill Klaassen	4773 1816
Treasurer	Janelle Mains	4779 4047	Contact Person	Jean Paterson	4775 2787

NEUROLOGICAL ALLIANCE AUSTRALIA (NAA) POSITION STATEMENT ON NDIS

Background:

The Neurological Alliance Australia (NAA) is an alliance of not-for-profit peak national organisations representing adults and children living with progressive neurological and neuromuscular diseases in Australia. The Alliance was established in 2010 to promote improved quality of life, coordinated services and greater research investment. The Alliance represents over 500,000 Australians living with progressive neurological or neuromuscular conditions that have no certain cure. This group includes adults and children, carers, families, friends and workmates whose life is, or has been, affected by a progressive neurological condition. The impact of progressive neurological conditions on people and families can undermine their resilience that is needed to remain purposeful and in control of their lives and prevent financial and emotion burden.

Progressive neurological and neuromuscular diseases are a set of complex and disabling conditions. While this broad group contains conditions with various characteristics, different disease trajectories and life expectancy all are degenerative and incurable. This results in significant disability, grief and need for expert care and personal assistance.

The introduction of a national disability insurance scheme (NDIS) has the potential to transform the lives of people living with progressive neuromuscular and neurodegenerative diseases through responsive funding to enable people to access services to allow for incremental support as the person's circumstances change.

The proposed NDIS recommends:

- early intervention for people with progressive neurodegenerative disease
- an *ongoing* assessment process that can anticipate *and respond to changing need*
- effective protocols for timely and smooth referrals to and from the different sectors
- easy access to quality disease specific information
- support for carers to promote wellbeing, and informal supports to alleviate carer burden.

These recommendations, if accepted, will go a long way in providing an appropriate support structure for people in need of urgent and sometimes complex support services following a diagnosis of a progressive neurological or neuromuscular disease. Early intervention will assist people diagnosed with this group of diseases to plan ahead, maintain employment and to remain as independent as possible for as long as possible. There are significant costs over a lifetime for many of this cohort and there is evidence to support the notion that with small, timely and targeted financial and social supports earlier in the disability this may help to retain relationships, sustain informal care and social supports which once lost are hard to recapture as the disease progresses.

Neurological Alliance Australia believes:

- An NDIS offers a significant solution to the chronic unmet need for early intervention and lifetime care and support services
- People diagnosed with rapidly progressive neurological disease must be eligible for and have access to NDIS services
- The collaboration of organisations involved in Neurological Alliance Australia and their commitment to best customer outcomes will play a vital role within an NDIS with respect to specialist information and education and expert individualised and personalised support and services

Neurological Alliance Australia calls for:

1. **Ongoing engagement with the NDIS to inform the design of the scheme through the provision of expert information on:**

a. needs of people with a Progressive Neurological and Neuromuscular disorder
b. the extensive body of evidence (research & evaluation, pilots and practice examples) in the sector
c. roles of not for profit disease specific organisations under an NDIS in the provision of information, education and training, volunteer support, assessment and referral

2. Eligibility from diagnosis to support early intervention to reduce the economic and social burden and improve quality of life

3. Disease sensitive and ongoing assessment untaken by experienced practitioners

a. Assessment tools must be sensitive to the diversity of the groups/clients
b. Assessors must have the right assessment tools – sensitive to the fluctuations, progression and "hidden symptoms"
c. Assessors must be trained and informed around specific disease processes and diversity within groups – not all the same
d. Initial and ongoing review driven by disease process and changing need

4. Seamless anticipatory care based on assessed needs for people affected by progressive neurological conditions throughout their life journey

a. Pathways of Care
b. Cross sector engagement and funding to facilitate timely, coordinated inter/multidisciplinary care and to reduce duplication and crisis management
c. Improved interface between the disability and ageing sectors to ensure people diagnosed with rapidly progressive neurological diseases when over the pension age have equal access to needs based care and support

Considered and endorsed by the Neurological Alliance Australia December 2012:

- Alzheimer's Australia
- Brain Injury Australia
- Friedreich Ataxia Research Association Australasia
- Huntington's Australia
- MND Australia
- Multiple Sclerosis Australia
- Muscular Dystrophy Australia
- Muscular Dystrophy Foundation
- Parkinson's Australia
- Spinal Muscular Atrophy Australia

Prana Biotech Publishes Huntington's Disease Animal Model Data for PBT2

This article is courtesy of hdbuzz.net

Prana Biotechnology has released data demonstrating their drug, PBT2, is effective in animal models of HD

By Dr Jeff Carroll on January 14, 2013 Edited by Dr Ed Wild

The Huntington Study Group and Prana Biotechnology are currently running a clinical trial, Reach2HD, to determine whether the drug PBT2 is effective in HD patients. Now, they've released the preclinical data behind the trial, showing the drug is effective in two animal models of HD.

History of PBT2

Many Huntington's disease families have been excited by word of a new player developing a novel treatment for HD. Prana Biotechnology, a drug development company in Australia, has developed a new drug they call **PBT2**.

PBT2 aims to prevent copper from sticking to the mutant huntingtin protein - a possible step on the road to HD.

HDBuzz has previously written about Prana and their drug, which works in a surprising and novel way. While all the details are not understood, the drug is designed to interfere with interactions between the huntingtin protein and the metal copper.

Interfering with copper in the body may sound like a strange and surprising way to attack Huntington's disease, but there is a history of investigating changes in copper in the brain of HD patients.

Another genetic disease called **Wilson's disease** is caused by mutations in a gene that helps cells get rid of excess copper. The cells of patients with Wilson's disease accumulate too much copper because they don't know how to get rid of it, thanks to their defective gene.

It turns out that Wilson's disease patients have brain damage in the same areas of the brain as Huntington's disease patients, and that in HD, these parts of the brain accumulate copper too. This supports the idea that copper might be important for the particular parts of the brain that die in HD.

Based on in-house work that suggested PBT2 was effective in Huntington's disease, Prana Biotechnology began working with the Huntington Study Group to initiate a trial of their drug in human HD patients. The trial, currently running in the US and Australia, is called **Reach2HD**.

This trial happened so fast that few people outside the company had seen the data that suggested their drug was effective. They've now published this data for everyone to see in the new Journal of Huntington's Disease.

The animal models

Before testing a drug in humans, scientists like to have an idea of whether it is safe and effective. The only way to study this is to give the drug to animals who have been genetically modified to carry the same mutant HD gene as human patients.

These animals have problems that mimic, in some ways, those experienced by HD patients. While the animals don't have Huntington's disease, they do provide an objective way of testing whether a drug has an impact on the problems caused by expression of the mutant HD gene.

To test PBT2, the team of scientists, lead by Stephen Massa of the University of California at San Francisco, turned to two different animal models of HD. First, they used a tiny worm with a big name - **'Caenorhabditis elegans'**. Unlike humans, with their billions and billions of brain cells, **C. Elegans** has precisely 302 brain cells.

Forcing **C. elegans**' worms to express a gene similar to the one that causes Huntington's disease in people causes these worms to become paralyzed and unable to move. Because the worms are so small and have a very short lifespan, they can be used to quickly test whether a drug reduces the harm associated with the mutant gene.

The second animal used to investigate the effectiveness of PBT2B was a mouse that has been genetically engineered to express a mutant HD gene. This gene makes them very sick, very fast - they have problems with coordinating their movements, show shrinkage in the brain similar to that seen in HD patients and ultimately die very young. These mice provide a simple tool for testing a Huntington's disease drug - scientists can simply give the mice a drug and see if it can improve any of their symptoms.

The results

In the worm model, PBT2 was very effective - worms treated with PBT2 were able to live for much longer without becoming paralyzed. Rescuing worms is nice, but it's a long way from people! The mice, despite being small and having fairly simple behaviors, are much closer to people. How did PBT2 do in HD mice?

While alive, HD mice treated with PBT2 showed some improvements in the coordination of their movements - that is, they were slightly less clumsy. More interestingly, treatment with PBT2 prolonged the survival of HD mice by a significant

amount: mice treated with the drug lived about 26% longer than untreated mice. That's a pretty decent extension, though we should remember that the mice were still quite sick during the extended period of their life.

Other measures were improved by PBT2 treatment as well. Like many HD patients, these HD mice lose weight. Weight loss can be a major problem for HD patients, and is difficult to combat. Treatment with PBT2 helped HD mice maintain body weight in a fairly dramatic fashion.

In the brain, HD mice showed shrinkage similar to that experienced by HD patients. This loss was significantly, but not completely, rescued by treating the mice with PBT2. This suggests the drug isn't just masking symptoms, but might actually be stopping the brain cell death that causes symptoms to occur.

Caveats and questions

All in all, it's easy to see why these scientists were excited about the results of PBT2. The beneficial effects in the mice, in particular, are pretty impressive.

As with any trial conducted in animals, it's worth thinking about the limitations. The mice, for example, were treated with PBT2 from 3 weeks of age - essentially from when they first start eating and drinking on their own, rather than nursing from their mothers. This is not what will happen in people, who are only being given the drug after their symptoms start. Can PBT2 work, even if it's only given when someone is already sick? We just don't know yet.

PBT2 has advantages over some other experimental drugs in HD. For one, it is known to get into the brain, where it needs to be to work. Furthermore, it has already been shown to be well tolerated in human Alzheimer's Disease patients, making it less likely that the drug will fail because of side-effects.

The clinical trial currently investigating PBT2 in HD patients is formally only designed to study whether the drug is safe in HD patients when administered for 26 weeks. But the investigators are also measuring a host of changes in these patients caused by HD, including behavior changes, thinking problems and biological changes in the blood, urine and brain. Looking at these things now may give us a hint of whether PBT2 is effective.

Especially in light of these positive results in animal models, HDBuzz is encouraged to hear that the trial is now fully recruited, and we look forward to hearing the results.

Ten Golden Rules for Reading a Scientific News Story

This article is courtesy of hdbuzz.net

Avoid the hype: HDBuzz presents ten 'golden rules' for reading a news story or press release about Huntington's disease

By Dr Ed Wild on September 05, 2011 Edited by Dr Jeff Carroll

Real progress is being made on the road to Huntington's disease treatments, but sometimes it feels like scientists promise more than they can deliver. So, HDBuzz has come up with ten 'golden rules' to help you decide whether a news story or press release offers genuine promise for HD, or whether its claims should be taken with a pinch of salt.

Snowflakes and glaciers

HDBuzz loves science. In our more philosophical moments, we like to imagine all the world's scientific research as a flurry of snowflakes, gently settling on a mountain top and gradually, over months, years, and decades, compacting into a huge, unstoppable glacier that can carve entire mountains.

No single snowflake could do that, but combined, over time, the power of science to change the world - and improve the lives of people with HD - is immense.

How science reaches the public

Science becomes 'official' when an article about a piece of research is published in a peer-reviewed scientific journal. But a lot of science reaches the public through press releases.

Increasing competition over scarce funding means that getting results published in a scientific journal may not be enough for scientists to keep their work going.

The agencies that fund science take their lead from the public, so one way for researchers to secure funding is to get the public excited about their research. So when a piece of work has so far only focused on a small area, one way to excite people is to get them to imagine the whole glacier, rather than just the snowflake.

Like a glacier, science moves slowly but can move mountains. Don't be fooled by anyone suggesting that a single snowflake can do the same.

So, universities and research companies have press offices, whose job is to encourage scientists to produce press releases, in which they often speculate about what applications their work may have, down the line.

Of course, part of what science is for is to come up with real-world uses for new discoveries. But it's a two edged sword, because many things that **might** happen, never do.

Another layer of speculation can get added, when press releases are turned by bloggers and journalists into news stories. Writing about big breakthroughs in common diseases gets more clicks and sells more papers than writing about small progress and obscure conditions.

What's the harm?

The result can be that press releases and news articles sometimes end up promising things that the scientific research could never deliver - or which are much further away than an article suggests.

This isn't the fault of the individual scientists, or of the press office, or the bloggers or journalists, or of the people reading the stories. Nobody sets out to mislead - but sometimes that can be the outcome, and it's bad news because it can lead to disappointment and loss of hope.

Ten Golden Rules

The good news is that disappointment can be avoided if readers know what to look out for.

So, HDBuzz has come up with Ten Golden Rules for reading a press release or scientific news article. They're here to help you to draw hope from scientific news where it's warranted - and avoid being let down where it's not.

1. **Be skeptical of anyone promising a "cure" for HD** now, or in the near future.
2. If something sounds **too good to be true**, it probably is.
3. Has the research been **published in a peer-reviewed scientific journal**? If not, the press release may not be much more than speculation.
4. Ask yourself whether the press release is announcing the **results of a project** - or just the start of the project, a new partnership or funding approval. There's a big difference.
5. The only way to show that something works in HD patients is to **test it in HD patients**.
6. A positive result in an **animal model of HD** is a very good start - but can't be called a cure - and plenty of things that work in mice fail when tested humans.
7. Something that **hasn't been tested in an HD animal model** has a very long way to go to become a treatment.
8. **Your mind is like a house** - it's good to keep it open, but if you leave it wide open, you never know who'll walk in.
9. Not sure about something you've read? **Ask HDBuzz to write about it**!
10. Finally, remember that **every day, science moves us towards effective treatments** for HD. Even negative results and treatment failures help us to focus on more fruitful ideas.

"The good news is that disappointment can be avoided if readers know what to look out for."

An example - 'block and replace' gene therapy

Recently, a story headed “Molecular Delivery Truck Serves Gene Therapy Cocktail” appeared on news site Science Daily. Similar articles appeared on many other sites, all reporting on work led by Prof R Jude Samulski of the University of North Carolina, and published in the journal PNAS.

The news article revealed that Samulski's team had done something pretty impressive. The research centered on a disease called alpha-1-antitrypsin deficiency - 'alpha-1' for short.

People with alpha-1 develop liver problems, because they have two faulty copies of a gene that tells cells how to make the alpha 1 protein. Part of the problem is that the healthy protein is missing, and part of the problem is that the mutant protein made by cells is harmful.

Samulski's group used a form of 'double-barreled' gene therapy to fix this problem in mice with the same genetic problem. First, they made a DNA-like molecule that would block production of the abnormal protein - a form of gene silencing. Then, they added a replacement gene that would be used by cells as a recipe for making the healthy protein.

They packaged these two payloads into a virus called AAV, which attaches to cells and injects its contents into them. Mice treated with the virus restored healthy levels of the alpha-1 protein and didn't develop liver problems.

Great work - shame about the press release

Let's be clear - this is great science and an innovative approach to a devastating illness. So what's the problem?

Use our ten golden rules to protect you against hype and disappointment.

Well, this research came onto our radar because the news reports about it all mentioned the potential of the approach for treating other 'protein folding' diseases like “cystic fibrosis, **Huntington’s Disease**, amyotrophic lateral sclerosis … and Alzheimer's disease”.

The news stories said that, because that's what was said in a press release by the researchers themselves, and again in the PNAS article.

The trouble is, the research didn't directly involve any of those other diseases - and huge obstacles stand in the way of it working in Huntington's disease or the other conditions mentioned. But you wouldn't necessarily know that from reading the news stories.

In the case of HD, there are two main problems. The first is that the huntingtin protein that causes HD is huge - seven times larger than the alpha-1 protein. The AAV virus is just too small to deliver a replacement huntingtin gene. Other viruses might be able to, but they're not as good at delivering the cargo into cells. The other problem is that once the alpha-1 protein has been made, it's released into the bloodstream, which means that a little goes a long way. Huntingtin protein, on the other hand, does all its work (and damage) inside cells - so any gene therapy needs to get into lots more cells in order to be beneficial.

The result of these problems is that the approach - ingenious though it is - simply can't be applied to HD now, and even if it were radically altered, it's unlikely it'll benefit HD patients for at least a decade - if at all.

You might think you have to know all about gene therapy to be able to spot these problems in applying it to HD.

In fact, there are enough clues there to enable non-scientists to treat this particular breakthrough with caution, even though it might have popped up in a Google news alert for “Huntington's disease”.

Using the golden rules

Applying our golden rules to this particular press release causes several alarm bells to ring.

Rule 2. The press release suggests that this one approach could be useful for five different, major diseases - sounds amazing … could it be **too good to be true**? Proceed with caution.

Rule 5. Tested in HD patients? No, this research only went as far as mice.

Rules 6 and 7. What about an **HD animal model**? Nope, the mice were models for alpha-1 deficiency, not Huntington's disease.

So you don't have to be an expert in the science of gene therapy for our rules to provoke some healthy scepticism about this particular press release.

That's where rules 8 and 9 come in - keep an open mind but remain cautious about breakthroughs - and if you read something you're not sure about, feel free to ask HDBuzz to investigate - either by emailing editor@hdbuzz.net or using the suggestion form at HDBuzz.net.

Rule ten

Rule ten is our favourite - because it brings us back to waxing lyrical about the snowflakes and the glacier. Rule ten is there to remind us that - whatever a particular bit of news can or can't tell us about the search for effective treatments for Huntington's disease - we're a bit closer today than we were yesterday, and tomorrow we'll be closer still

DONATIONS TO HUNTINGTONS QUEENSLAND

If you would like to donate to Huntingtons Queensland, please return the slip below to our office with your payment. Alternatively you can donate online – go to www.huntingtonsqld.com . All donations over $2 are tax deductible and we will send you a receipt for taxation purposes.

Personal Details:

Title..... Name.....

Address.....

.....Postcode.....

Telephone..... Mobile..... Fax.....

Email *(please print clearly)*.....

Payment Options:

Cash ☐ Cheque ☐ Money Order ☐ Visa ☐ MasterCard ☐ Visa Debit ☐ MasterCard Debit ☐

Card No _ _ _ _ / _ _ _ _ / _ _ _ _ / _ _ _ _ Expiry Date _ _ / _ _ CVC: _ _ _ AMOUNT $.....

Name on Card ..... Signature ..... Date.....

Thank you so much for your very kind donation to Huntingtons Queensland! We will post you a tax receipt. Please retain a copy of your completed slip for your records and mail, fax or scan & email a copy to:

Huntingtons Queensland PO Box 635 Annerley Qld 4103
Tel: (07) 3391 8833 Fax: (07) 3391 0443 Email: admin@huntingtonsqld.com

FUND RAISING

FINANCIAL ASSISTANCE TO HUNTINGTONS QUEENSLAND

We have received and gratefully acknowledge major financial assistance from the following kind donors:

Don &Barb Gray
J&L Hartkopf
Z Walker
R Ryan
William van Heel
Joyce Bennett
Paul & Meg Johnson
Jody Williams
J Cope
AN Longland
D Hewitt
Jack Flitcroft
Joy McInerney
DA Harrison
Nicole Lofting
M Lupton
Diana Leonard (Circle of Friends)
Dept of Housing Public Works (George Street Staff)
Darren Wright (Aidacare)
Alwyn Peffer & Company (Management & Staff)
Skinner Family (Brad, Rita, Jim, Fiona, Ken & Meredith)

HUNTINGTONS QUEENSLAND NOMINATED AS BENEFICIARY

Our sincere thanks continue to **Beecham Holden Caboolture** who has kindly nominated Huntingtons Queensland as the beneficiary for a charitable donation by way of CTP on first time registered vehicles sold through them.

You can contact them on:

Ph: 1300 661 958
29 Bribie Island Road Caboolture

GENEROSITY

For a number of years now Miss Alethea Harding-Smith has been a generous benefactor of Huntingtons Queensland. In this difficult economic climate her continued support of our much needed services is very much appreciated by our clients, their families and carers, the management committee and staff.

DONATIONS TO HUNTINGTONS QUEENSLAND

If you would like to donate to Huntingtons Queensland and have internet access, go to our website www.huntingtonsqld.com. Scroll down to the 'Please Make a Donation' section on the bottom left, click on the button <CLICK HERE> and follow the instructions.

Alternately you can return the slip on page 11 of this Newsletter and return to us with your donation – cheque, money order or credit card.

All donations over $2 are tax deductible and we will send you a receipt for taxation purposes.

QUT STAFF COMMUNITY WELFARE FUND

We'd like to express our sincere thanks to the kind Staff at QUT for their recent donation which has allowed us to update our crockery and cutlery in the Day Centre. This includes special adaptive items such as cutlery, plates and specialised drinking cups.

QUT's Executive Director of Finance & Resource Planning, Stephen Pincus, presenting the cheque to our Executive Officer, Cheryl Miller.

FLORENCE DANNELL HOUSE

You may or may not know why our Annerley facility is named Florence Dannell House. Florence Dannell left a generous bequest to the Association in the 1990s. It was her kind gesture that enabled the purchase, re-design and restoration of our building.

Bequests enable us to continue and expand the provision of the important support and services we deliver. All donations and bequests, no matter how small, are gratefully received and applied to the work we do.

If you, a family member or business would like to contribute please feel free to contact Cheryl Miller or Anne Stanfield for further details on 07 3391 8833, cheryl@huntingtonsqld.com or anne@huntingtonsqld.com

THE ROTARY CLUB OF BRISBANE INNER WEST

Our thanks to the Rotarians of Brisbane Inner West for conducting their Christmas raffle.

Half the proceeds ($925) of all tickets that our members and staff sell on their behalf have been donated back to Huntingtons Queensland.

So many thanks to those members of our Association, management committee members and staff who took time out to sell tickets.

HUNTINGTONS QUEENSLAND

Florence Dannell House
385 Ipswich Rd cnr Ponsonby St
Annerley Brisbane

All Correspondence to
PO Box 635
Annerley Q 4103

Phone: (07) 3391 8833
Fax: (07) 3391 0443

Emails:
cheryl@huntingtonsqld.com
admin@huntingtonsqld.com
christine@huntingtonsqld.com
theressa@huntingtonsqld.com
fiona@huntingtonsqld.com
anne@huntingtonsqld.com

Website: www.huntingtonsqld.com

Huntingtons Queensland
is a not-for-profit service organisation.
Established in 1976.

Our Mission is:

To provide professional support and advocacy for all persons affected by Huntington's Disease in Queensland.

Our Services Include:

- Providing individual and family support
- Facilitating the HD Day Respite Program
- Facilitating support group meetings
- Recreational activities for families with young children
- Organising respite holidays
- Providing information to families and health professionals
- Distributing a regular Newsletter
- Co-ordinating the annual HD Awareness activities
- Fundraising activities

Management Committee 2012/2013:

President	Gerry Doyle
Vice President	Robert Westley
Secretary	Pam Cummings
Treasurer	Heather Whye
Committee Members:	Jan Szlapak
	Alan McKinless
	Marty Harmsworth
	Shirley Ross
	Emma Kyle
	Gwen Pratten

Staff Members:

Executive officer	Cheryl Miller
Senior Welfare Officer	Christine Fox
Welfare Officer	Theressa Byrne
Welfare Officer	Fiona Kerr
Administration Manager	Anne Stanfield
Telemktg / Admin Assistant	Helen Johnston
Bookkeeper	Jan Mealy

CONTRIBUTIONS & DISTRIBUTION

Please feel free to submit articles or photographs for selection for publication in this Newsletter. The deadline for the next issue is 15th May 2013. Please email or post articles, details above. Please be aware that the Newsletter is published on *www.huntingtonsqld.com* in addition to postal and email distribution.

This Newsletter has been printed free of charge by the office of Graham Perrett, Federal Member for Moreton. Our kind thanks to Graham & Staff.